

NIH Burden of Muscle Disease Workshop
Jan. 26 & 27, 2005
Lister Hill Auditorium, NIH Campus
Bethesda, MD

DRAFT AGENDA

Wed. Jan. 26

7:00-8:00 am Registration

8:00 am **Opening Remarks**
Stephen I. Katz, MD, PhD, *Director of the National Institute of Arthritis
and Musculoskeletal and Skin Diseases*

I. Introductory Session

Moderators: Richard Lymn, PhD, *NIAMS/NIH*
Richard Moxley, III, MD, *University of Rochester*

8:05 am **Goals of the Workshop**
Richard Lymn, PhD, *NIAMS/NIH*

8:15am **Defining Chronic Muscle Disease**
Craig McDonald, MD, *University of California, Davis*

8:25 am **Muscular Dystrophies – a Paradigm for Chronic Myopathies**
Richard Moxley, III, MD, *University of Rochester*

8:35 am **Assessing Burden of Illness**
James A. Schuttinga, PhD, *NIH Office of Science Policy and Planning*

8:45 am **Approaches to Burden of Illness in Canada**
Michael Wolfson, PhD, *Statistics Canada*

8:55-9:45 am Questions and Discussion

9:45-10:00 am Break

II. Personal and Family Factors

Moderators: Craig McDonald, MD, *University of California, Davis*
Jerry R. Mendell, MD, *Ohio State University*

10:00 am **Introduction**
Craig McDonald, MD, *University of California, Davis*

10:10 am **Challenges to Education and Careers**
Steven James Tingus, M.S., C. Phil., *Director, National Institute on
Disability and Rehabilitation Research*

- 10:20 am **Parental Perspectives**
Pat Furlong, *Parent Project Muscular Dystrophy*
- 10:30 am **Multi-Generational Aspects**
William G. Michael, CPA, *Facioscapulohumeral Muscular Dystrophy Society*
- 10:40 am **Views on Disease Management of Myotonic Dystrophy**
Margaret Bowler, *Myotonic Dystrophy Support Group, UK*
- 10:50 am **Myotonic Dystrophy Support Groups in the United States**
Shannon Lord, Atlanta, GA
- 11:00-12:00 pm Questions and Discussion
- 12:00-1:00 pm Lunch

III. Health and Psychosocial Burdens

- Moderators: Susan Iannacone, MD, *University of Texas Southwestern Medical Center*
John Day, MD, PhD, *University of Minnesota*
- 1:00 pm **Measures of Caregiver and Family Stress**
Veronica Hinton, PhD, *Columbia University*
- 1:10 pm **Quality of Life and Social Issues in Myotonic Dystrophy**
Cynthia Gagnon, M.Sc., *Carrefour de santé de Jonquière*
- 1:20 pm **Measuring Quality of Life (QoL) in Adolescents with Disabilities and Chronic Conditions**
Tari D. Topolski, PhD, *University of Washington*
- 1:30-1:45 Questions and Discussion
- 1:45 pm **Health Related Quality of Life and Functional Status**
James W. Varni, Ph.D., *Texas A&M University*
- 1:55 pm **Disease-Specific QoL Instruments - Arthritis**
Deborah P. Lubeck, PhD, *Amgen Inc.*
- 2:05 pm **QoL: Issues for Persons with Neuromuscular Diseases**
Ted Abresch, MS, *University of California, Davis*
- 2:15 pm **Burden of Adult Muscle Diseases**
Michael Rose, MD, *King's College, London*
- 2:25- 2:40 pm Questions and Discussions
- 2:40 pm **Advances in Health Outcomes Measurement**
Bryce Reeve, PhD, *National Cancer Institute/NIH*

2:50 pm **Patient-Reported Outcomes Measures**
Deborah Ader, PhD, *NIAMS/NIH*

3:00-3:15 pm Break

IV. Epidemiologic Measures of Chronic Myopathies

Moderators: Ed Brann, MD, MPH, *Center for Disease Control and Prevention (CDC), Atlanta*
Paula Clemens, MD, *University of Pittsburgh*

3:15 pm **Sources of Epidemiologic Data**
Aileen Kenneson, PhD, *CDC*

3:25 pm **Muscular Dystrophy Surveillance Tracking and Research Network (MD STARnet)**
Paul Romitti, PhD, *University of Iowa*

3:35 pm **Duchenne Muscular Dystrophy Survey**
Eric Henricson, MPH, *Children's National Medical Center, Wash. DC*

3:45 pm **National Registry for Myotonic Dystrophy and Facioscapulohumeral Dystrophy Patients and Families**
Richard Moxley, III, MD, *University of Rochester*

3:55-5:00 pm Questions and Discussion

Thurs. Jan. 27

V. Cost Measures of Chronic Myopathies

Moderators: Scott Grosse, PhD, *CDC*
James A. Schuttinga, PhD, *NIH Office of Science Policy and Planning*

8:00 am **Components and Measures of Health Care Costs**
Ted Miller, PhD, *Pacific Institute for Research and Evaluation*

8:10 am **Economic Impact of Spina Bifida**
Norman J. Waitzman, PhD, *University of Utah*

8:20 am **Private Insurance Claims Data Specific to Muscular Dystrophy**
Scott Grosse, PhD, *CDC*

8:30 am **Hospital Discharge Datasets and the Cost of Inpatient Admissions**
Claudia Steiner, *AHRQ*

8:40 am **Family Out-of-Pocket Expenses for Children with Disabilities**
Paul W. Newacheck, DrPH, *University of California at San Francisco*

8:50 am **Caregiver Time Costs for Patients with Spina Bifida**
John M. (Mick) Tilford, MD, *University of Arkansas*

9:00-10:00 am Questions and Discussion

10:00-10:15 am Break

VI. Preference-Based Summary Measures of Health

Moderators:

William Lawrence, MD, MS,
Joanna Siegel, ScD, RN, *Agency for Healthcare Research and Quality (AHRQ)*

10:15 am **Quality-Adjusted Life Years (QALYs)**

William Lawrence, MD, MS, *AHRQ*

10:25 am **Types of Instruments and Use of Proxies-Health Utilities Index**

William J. Furlong, MSc, *McMaster University, ON, Canada*

10:35 am **Population or Patient Preferences for Health State Weights**

Joanna Siegel, ScD, RN, *AHRQ*

10:45 am **Disability-Adjusted Life Years (DALYs) in US Burden of Disease and Injury Study**

Catherine M. Michaud, MD, PhD, *Harvard University*

10:55 am **DALY Measurements in Australia**

Armineh Zohrabian, PhD, *CDC*

11:05-12:00 pm Questions and Discussion

12:00-1:00 pm Lunch

VII. Lessons and Opportunities for Measuring Impact of Muscle Diseases

(Open discussion among workshop attendees)

Moderators: Ed Brann, MD, MPH, *CDC*

Richard Moxley, III, MD, *University of Rochester*

1:00-1:30 pm **Available Data Sets and Data Needs- Prevalence and Incidence**

1:30-2:00 pm **Data Assessment - Strategies for New Information or Analysis**

2:00-2:30 pm **Use of Burden Measurements in Assessing Effectiveness of Treatment**

2:30-3:00 pm **Current Estimates of the Burden of Muscle Diseases**

3:00 pm Meeting Adjourned